U.S. FOOD & DRUG ADMINISTRATION

CENTER FOR DRUG EVALUATION AND RESEARCH

Advancing Health Through Innovation:

New Drug Therapy Approvals 2024

INNOVATION | PREDICTABILITY | ACCESS

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Table of Contents

Director's Message

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Director, Center for Drug Evaluation and Research

Welcome to FDA's Center for Drug Evaluation and Research's (CDER) 14th annual report, *Advancing Health Through Innovation: New Drug Therapy Approvals*. The 2024 New Drug Therapy Approvals report is designed to showcase CDER's role in bringing safe and effective drugs to market for patients and consumers.

This report describes CDER's 2024 notable drug approvals, which are actions we consider likely to have a significant impact on patient care and public health. These include novel drug approvals that contain a new active ingredient not previously approved by FDA. Also included are previously approved active ingredients that were also approved in 2024 to treat a different condition or new patient population.

Throughout the report, we describe new therapies CDER approved in 2024 for safety and efficacy, as well as the regulatory tools used to streamline the review and approval of these applications. We approved most of these new treatments on or before their goal dates as defined by congressionally authorized agreements with industry (referred to as user fee programs). More than half of these drugs were approved in the U.S. before our regulatory counterparts in other countries did so.

CDER's work on biosimilar biological products (biosimilars) reached several major milestones in 2024. CDER approved a record total of 18 biosimilars for 8 reference products in 2024, more than any previous year. We also surpassed our 60th biosimilar approval since 2015 when the first biosimilar was approved. In addition, this year 9 biosimilars, including those approved in 2024 and in previous years, were approved as interchangeable biosimilars, which means that they may be substituted for their reference product at the pharmacy without the intervention of the prescriber, subject to state law, similar to how generic drugs are substituted for brand name drugs.

Our 2024 drug approvals include therapies for a variety of diseases and conditions, including rare diseases. FDA approved a significant number of rare disease therapies with orphan drug designation for patients with few to no treatment options. Therapies with orphan drug designations accounted for over 50% of our novel drug approvals, highlighting our steadfast commitment to address the huge unmet medical need in rare diseases. This year, we approved 26 novel therapies with this designation, including two of which treat conditions with no previously approved treatment such as Neimann-Pick disease type C and WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis).

This report is intended to feature CDER's 2024 notable approvals. FDA's Center for Biologics Evaluation and Research (CBER) also approves novel biologics and there are times when CDER and CBER may collaborate on certain approval actions. For more information on CBER's 2024 product approvals, please visit CBER's webpage for 2024 Biological Approvals.

We hope this report illustrates CDER's hard work and unwavering commitment to advance patient care through the timely and thorough review and approval of safe and effective treatments.

Patrizia Cavazzoni, M.D.
Director, Center for Drug Evaluation and Research

Executive Summary

CDER approved many safe and effective drug therapies in 2024. These approvals, spanning a broad scope of diseases and conditions, aim to help many people live better and potentially longer lives.

Innovation Across Medical Conditions

In 2024, we approved 50 new drugs never before approved or marketed in the U.S., known as “novel” drugs. We also made other important approval decisions, such as approving previously approved drugs for new uses and broader patient populations.

The 2024 actions, both novel and other important drug approvals, focus on prevention, diagnosis, and treatment of diseases and conditions such as:

- Infectious diseases and associated conditions, including COVID-19, bloodstream infections, bacterial skin and associated tissue infections, community-acquired bacterial pneumonia, and urinary tract infections.
- Neurological conditions, such as Alzheimer’s disease and multiple sclerosis.
- Opioid use, misuse, and abuse.
- Heart, blood, and kidney diseases, including hypertension (high blood pressure); types of hemophilia (a blood clotting disorder); and major adverse cardiovascular events in adults at high risk.
- Lung diseases, such as chronic obstructive pulmonary disease.
- Different types of cancer, such as biliary, bladder, blood, breast, esophageal, lung, and low-grade gliomas (tumors that start in the brain).

New Drugs for Patients with Rare Diseases

Patients with rare diseases are often in critical need of new therapies, as these individuals generally have few or no existing treatment options. In 2024, 26 of 50, or 52% of our novel drug approvals were approved for rare diseases, including:*

- Neimann-Pick disease type C, a genetic disease that results in progressive neurological symptoms and organ dysfunction.
- Duchenne muscular dystrophy, a severe, inherited disease that causes progressive muscle weakness and muscle wasting.
- Primary biliary cholangitis, a chronic autoimmune disease that damages the bile ducts in the liver.
- Familial chylomicronemia syndrome, a life-threatening disease that prevents the body from digesting fats.
- Classic congenital adrenal hyperplasia, a group of genetic disorders that affect the adrenal glands and cause a deficiency in cortisol and/or aldosterone.

In 2024, CDER also approved many therapies for rare cancers or tumors, including:

- HER2-positive (IHC3+) tumors, which have higher levels of the human epidermal growth factor receptor 2 (HER2) protein that enables faster growth and spread.
- Grade 2 astrocytoma, a slow-growing brain tumor that can become more aggressive over time.
- Gastroesophageal junction adenocarcinoma, a rare type of cancer that starts in the gastroesophageal junction.
- Small cell lung cancer.

**Not all drugs for rare diseases receive orphan drug designation.*

Efficiencies in Bringing Therapies to Market

Our 2024 approvals demonstrate efficiencies in our review process, as shown by the following:

- **User Fee Goals Performance:** Of the 50 new drugs approved in 2024, CDER met or exceeded its Prescription Drug User Fee Act (PDUFA) goal dates for 47 of these approvals (94%).
- **First Cycle Approvals:** In 2024, CDER approved 37 of the 50 novel approvals (74%) on the first cycle. This differs from when CDER initially is unable to approve a drug because information in the application does not support approval. Subsequently, the sponsor resubmits the application with additional information, starting another review cycle that may lead to drug approval.
- **Approvals in U.S. Before Other Countries:** 34 of the 50 novel drugs approved in 2024 (68%) were first approved in the U.S.
- **Expedited Programs for Serious Conditions:** CDER has four broadly applicable programs to facilitate and expedite development and review of drugs for serious or life-threatening conditions: fast track designation, breakthrough therapy designation, priority review designation, and accelerated approval. In 2024, 33 of the 50 of CDER's novel drug approvals (66%) used one or more of these expedited programs, which helped bring new therapies to the market sooner.

CDER's Novel Drug Approvals of 2024

In 2024, CDER approved 50 novel drugs, either as new molecular entities (NMEs) under New Drug Applications (NDAs), or as new therapeutic biologics under Biologics License Applications (BLAs). The active ingredient(s) in a novel drug have not been approved in the U.S.

CDER's novel drug approvals for 2024 are listed alphabetically below by proprietary name.*

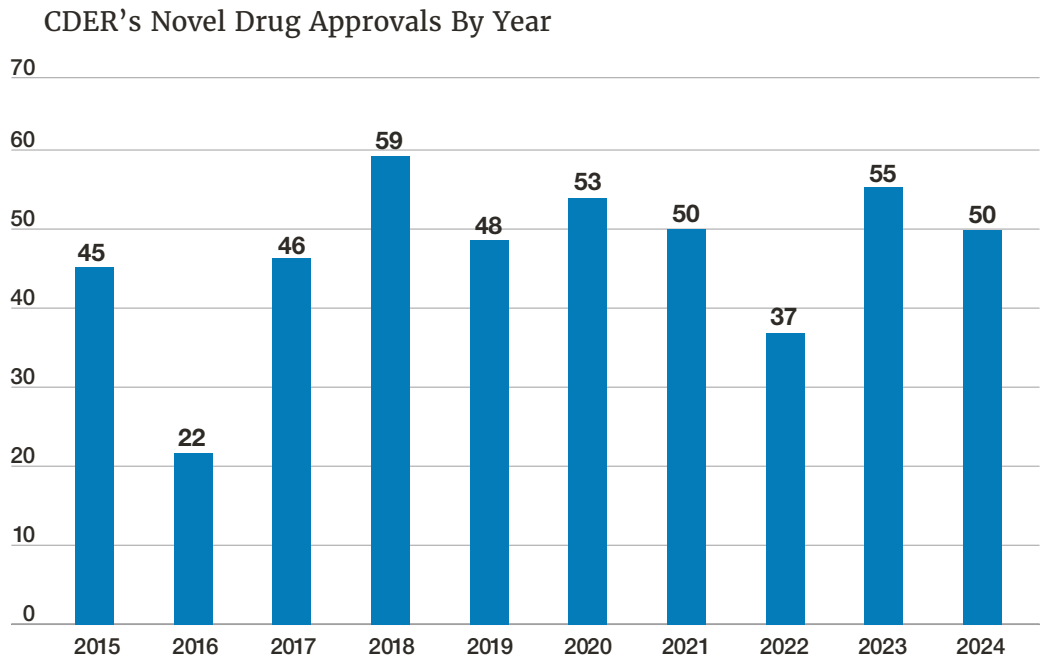
See Appendix B for a summary chart of designations for CDER's novel approvals.

Proprietary Name	Active Ingredient(s)
Alhemo	concizumab-mtci
Alyftrek	vanzacaftor, tezacaftor, deutivacaftor
Anktiva	nogapendekin alfa inbakicept-pmln
Aqneursa	levacetylleucine
Attruby	acoramidis
Bizengri	zenocutuzumab-zbco
Cobenfy	xanomeline, trospium chloride
Crenessity	crinecerfont
Duvyzat	givinostat
Ebglyss	lebrikizumab-lbkz
Ensacove	ensartinib
Exblifep	cefepime, enmetazobactam
Flyrcado	flurpiridaz F 18
Hympavzi	marstacimab-hncq
Imdelltra	tarlatamab-dlle
Iomervu	iomeprol
Iqirvo	elafibranor
Itovebi	inavolisib
Kisunla	donanemab-azbt
Lazcluze	lazertinib
Leqselvi	deuruxolitinib
Letybo	letibotulinumtoxinA-wlbg
Livdelzi	seladelpar
Lumisight	pegulicianine
Miplyffa	arimoclomol
Nemluvio	nemolizumab-ilto
Niktimvo	axatilimab-csfr
Ohtuvayre	ensifentrine
Ojemda	tovorafenib
Orlynvah	sulopenem etzadroxil, probenecid
Piasky	crovalimab-akkz
Rapiblyk	landiolol
Revuforj	revumenib
Rezdiffra	resmetirom
Rytelo	imetelstat
Sofdra	sofpironium
Tevimbra	tislelizumab-jsgr
Tryngolza	olezarsen
Tryvio	aprocitentan
Unloxcyt	cosibelimab-ipdl
Vafseo	vadadustat
Voranigo	vorasidenib
Voydeya	danicopan
Vyloy	zolbetuximab-clzb
Winrevair	sotatercept-csrk
Xolremdi	mavorixafor
Yorvipath	palopegteriparatide
Zelsuvmi	berdazimer
Zevtera	ceftobiprole medocaril sodium
Ziihera	zanidatamab-hrii

**This information is accurate as of December 31, 2024. In rare instances, CDER may need to change a drug's NME designation or the status of its application as a novel BLA. For instance, new information may become available that could lead to a reconsideration of the original designation or status. If CDER makes these types of changes, the agency intends to communicate the nature of, and the reason for, any revisions as appropriate.*

CDER's Annual Novel Drug Approvals: 2015 – 2024

The 10-year graph below shows that from 2015 through 2024, CDER has averaged about 47 novel drug approvals per year.



First-in-Class Drugs

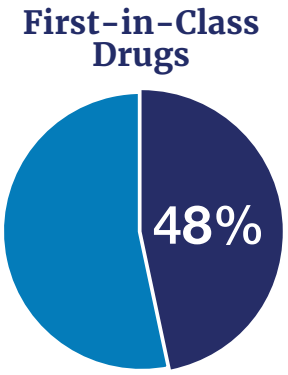
CDER identified 24 of the 50 novel drugs approved (48%) in 2024 as first-in-class. These drugs produce a novel pharmacologic effect, the impact or influence that a drug has on the body or a specific biological target, in a disease.

Novel drugs approved in 2024 that CDER identified as first-in-class were:

Anktiva, Aqneursa, Bizengri, Cobenfy, Crenessity, Duvyzat, Hympavzi, Imdelltra, Iqirvo, Lumisight, Miplyffa, Nemluvio, Niktimvo, Revuforj, Rezdiffra, Rytelo, Tryngolza, Tryvio, Voydeya, Vyloy, Winrevair, Xolremdi, Zelsuvmi, Ziihera

Notable examples of novel first-in-class approvals include:

- **Anktiva (nogapendekin alfa inbakicept-pmln)** intravesical solution (medications placed directly in the bladder through a catheter) to treat nonmuscle invasive bladder cancer with carcinoma in situ with or without papillary tumors that is unresponsive to prior therapy with Bacillus Calmette-Guérin.
- **Cobenfy (xanomeline and trospium chloride)** capsules are the first muscarinic-acting (mimics acetylcholine's action on muscarinic receptors) drug to treat schizophrenia in adults.
- **Lumisight (pegulicianine)** intravenous injection for fluorescence imaging in adults with breast cancer as an adjunct for the intraoperative detection of cancerous tissue within the resection cavity following removal of the primary specimen during lumpectomy surgery.
- **Nemluvio (nemolizumab-ilto)** for injection to treat adults with prurigo nodularis, a chronic skin disorder characterized by multiple, firm, flesh-to-pink colored pruritic papules, plaques, and nodules.



CDER identified 24 out of the 50 novel drugs (48%) approved in 2024 as first-in-class.

- **Revuforj (revumenib)** tablets and oral solution to treat relapsed or refractory acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in patients 1 year and older.
- **Rezdiffra (resmetirom)** tablets to treat adults with noncirrhotic MASH (metabolic-dysfunction associated steatohepatitis) with moderate-to-advanced liver fibrosis (consistent with stages F2 to F3 fibrosis). MASH is liver inflammation caused by excess fat cells. Rezdiffra was approved through the Accelerated Approval pathway.
- **Tryvio (aprocitentan)** tablets to treat hypertension (high blood pressure) in combination with other antihypertensive medications, to lower blood pressure in adults who are not adequately controlled on other medications.

Drugs for Rare Diseases

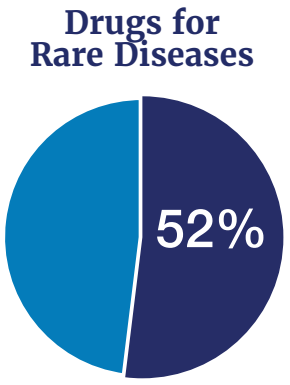
In 2024, 26 of CDER's 50 novel drug approvals (52%), were approved to treat rare or "orphan" diseases (diseases that affect fewer than 200,000 people in the U.S.). Patients with rare diseases often have few or no drugs available to treat their conditions.

Novel drugs approved in 2024 with the orphan drug designation were: *

Alhemo, Alyftrek, Aqneursa, Attruby, Bizengri, Crenessity, Duvyzat, Hympavzi, Imdelltra, Iqirvo, Livdelzi, Miplyffa, Niktimvo, Ojemda, Piasky, Revuforj, Rytelo, Tevimbra, Tryngolza, Voranigo, Voydeya, Vyloy, Winrevair, Xolremdi, Yorvipath, Ziihera

Examples of novel approvals of 2024 for rare diseases include:

- **Aqneursa (levacetylleucine)** oral suspension to treat neurological manifestations of Niemann-Pick disease type C in adults and pediatric patients weighing at least 15 kg. Niemann-Pick disease type C is a genetic disorder caused by variants in either the NPC1 or NPC2 gene, resulting in impaired intracellular transport of cholesterol and other lipids. The disease affects the nervous system and other organs and systems.
- **Crenessity (crinecerfont)** capsules and oral solution to treat classic congenital adrenal hyperplasia, a genetic disorder affecting the adrenal glands that causes a deficiency in certain hormones (cortisol and sometimes aldosterone) and excess of other hormones (androgens).
- **Duvyzat (givinostat)** oral suspension to treat Duchenne muscular dystrophy in patients 6 years of age and older. Duchenne muscular dystrophy is a genetic disorder characterized by progressive muscle degeneration and weakness.
- **Hympavzi (marstacimab-hncq)** subcutaneous injection to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients 12 years of age and older with hemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors, or hemophilia B (congenital factor IX deficiency) without factor IX inhibitors. Hemophilia is a disorder that prevents blood from clotting properly.
- **Imdelltra (tarlatamab-dlle)** for intravenous injection to treat adults with extensive stage small cell lung cancer with disease progression on or after platinum-based chemotherapy. Imdelltra was approved through the Accelerated Approval pathway.



Over half (52%) of the drugs CDER approved in 2024 received orphan drug designation.

- **Iqirvo (elafibranor)** tablets to treat primary biliary cholangitis in combination with ursodeoxycholic acid in adults who have an inadequate response to ursodeoxycholic acid, or as monotherapy in patients unable to tolerate ursodeoxycholic acid. Primary biliary cholangitis is a chronic autoimmune disease in which the liver's bile ducts become inflamed and slowly destroyed. Iqirvo was approved through the Accelerated Approval pathway.
- **Livdelzi (seladelpar)** capsules for the treatment of primary biliary cholangitis in combination with ursodeoxycholic acid in adults who have an inadequate response to ursodeoxycholic acid, or as monotherapy in patients unable to tolerate ursodeoxycholic acid.
- **Miplyffa (arimoclomol)** capsules as the first treatment for the neurological manifestations of Niemann-Pick disease type C, in combination with miglustat, in patients 2 years of age and older.
- **Ojemda (tovorafenib)** oral suspension to treat patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. A glioma is a tumor that originates in the brain or spinal cord from glial cells, which support and protect nerve cells. Ojemda was approved through the Accelerated Approval pathway.
- **Tryngolza (olezarsen)** injection to treat adults with familial chylomicronemia syndrome, a rare disease that prevents the body from digesting fats.
- **Voranigo (vorasidenib)** tablets for the treatment of patients 12 years and older with Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation following surgery including biopsy, sub-total resection, or gross total resection. An astrocytoma is a type of brain tumor that originates from star-shaped glial cells in the brain or spinal cord called astrocytes. An oligodendroglioma is a type of brain or spinal cord tumor that originates in glial cells called oligodendrocytes.
- **Voydeya (danicopan)** tablets as add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis in adults with paroxysmal nocturnal hemoglobinuria. Paroxysmal nocturnal hemoglobinuria causes destruction of red blood cells (hemolysis) by the complement system (part of the immune system) and some hemolysis may be extravascular (outside of blood vessels, such as in the spleen, liver, bone marrow, and lymph nodes).
- **Vyloy (zolbetuximab-clzb)** intravenous injection in combination with fluoropyrimidine- and platinum-containing chemotherapy for the first-line treatment of adults with locally advanced unresectable or metastatic HER2-negative gastric or gastroesophageal junction adenocarcinoma whose tumors are claudin 18.2 positive as determined by an FDA-approved test. Adenocarcinoma develops in glandular tissue.
- **Winrevair (sotatercept-csrk)** subcutaneous injection to treat adults with pulmonary arterial hypertension (WHO Group 1) to increase exercise capacity, improve WHO functional class, and reduce the risk of clinical worsening events. Pulmonary arterial hypertension is a chronic condition that causes high blood pressure in the arteries of the lungs.
- **Xolremdi (mavorixafor)** capsules for the treatment of WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis) in patients 12 years of age and older to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is a rare genetic disease that causes the body's immune system to not function properly, predisposing it to infections.

- **Yorvipath (palopegteriparatide)** subcutaneous injection to treat hypoparathyroidism in adults. Patients with hypoparathyroidism have abnormally low levels of parathyroid hormone, which leads to low levels of calcium in the blood and symptoms such as muscle cramping and spasms, nerve tingling, and fatigue.
- **Ziihera (zanidatamab-hrii)** intravenous injection for the treatment of adults with previously treated, unresectable or metastatic HER2-positive (IHC 3+) biliary tract cancer, a rare and aggressive type of cancer that affects the organs of the biliary system, which includes the gallbladder and bile ducts. Ziihera was approved through the Accelerated Approval pathway.

**Not all drugs for rare diseases necessarily receive orphan designation.*

Other Novel Drug Approvals

In addition to the first-in-class and drugs for rare diseases, CDER approved these notable approvals in 2024:

- **Exblifep (cefepime and enmetazobactam)** for intravenous injection to treat patients 18 years and older with complicated urinary tract infections, including pyelonephritis caused by the following susceptible microorganisms: *Escherichia coli, Klebsiella pneumoniae, Pseudomonas aeruginosa, Proteus mirabilis,* and *Enterobacter cloacae complex*. A complicated urinary tract infection occurs in patients with structural or functional abnormalities in the urinary tract, or certain signs or symptoms.
- **Flyrcado (flurpiridaz F 18)** intravenous injection for positron emission tomography (PET) myocardial perfusion imaging (MPI) in adults with known or suspected coronary artery disease to evaluate for myocardial ischemia and infarction. Myocardial ischemia occurs when the heart muscle does not receive enough blood flow and an infarction is tissue death that occurs when an area of the body doesn't receive enough blood flow.
- **Kisunla (donanemab-azbt)** intravenous injection to treat Alzheimer's disease, a brain disorder that gradually destroys memory and thinking skills and eventually the ability to perform everyday tasks. It is the most common cause of dementia in older adults, accounting for 60-70% of cases.
- **Lazcluze (lazertinib)** tablets, in combination with amivantamab, for the first-line treatment of adults with locally advanced or metastatic non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 L858R substitution mutations, as detected by an FDA-approved test. NSCLC is a type of cancer that forms in the lung tissues.
- **Orlynvah (sulopenem etzadroxil and probenecid)** tablets for the treatment of uncomplicated urinary tract infections caused by the designated microorganisms *Escherichia coli, Klebsiella pneumoniae,* or *Proteus mirabilis* in adult women who have limited or no alternative oral antibacterial treatment options. An uncomplicated urinary tract infection is a bacterial infection of the bladder that occurs in females without structural abnormalities of the urinary tract or other health conditions.

CDER approved a new amyloid beta-directed antibody to treat Alzheimer's disease in 2024.

- **Vafseo (vadadustat)** tablets for the treatment of anemia due to chronic kidney disease in adults who have been receiving dialysis for at least 3 months. Anemia (an insufficient number of red blood cells to carry oxygen throughout the body) is a common complication of chronic kidney disease, a long-term condition that occurs when the kidneys are damaged.
- **Zevtera (ceftobiprole medocaril sodium)** for intravenous injection for the treatment of adults with *Staphylococcus aureus* bloodstream infections (bacteremia), adults with acute bacterial skin and skin structure infections, and adults and children (3 months to less than 18 years old) with community-acquired bacterial pneumonia.

Innovation: Use of Expedited Development and Review Pathways

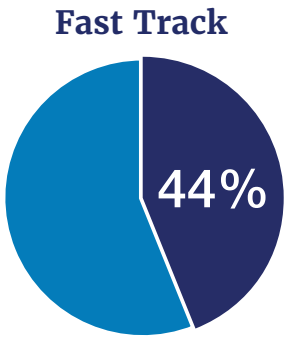
CDER used diverse regulatory approaches to enhance and expedite drug review in 2024. These approaches enable increased flexibility, efficiency, and interactions between CDER staff and drug developers. They often also allow shorter review times to speed the availability of new therapies to patients with serious conditions, especially in cases where there are no satisfactory alternatives, while preserving FDA's rigorous standards for safety and effectiveness.

Fast Track

CDER granted fast track status to 22 of the 50 novel drugs (44%) in 2024. Fast track speeds development and review of new drugs and biologics by increasing the level of communication between FDA and drug developers and by enabling CDER to review portions of a drug application on a rolling basis.

Drugs granted Fast Track status were:

Anktiva, Aqneursa, Bizengri, Crenessity, Duvyzat, Ebglyss, Exblifep, Kisunla, Leqselvi, Lumisight, Miplyffa, Niktimvo, Orlynvah, Revuforj, Rezdiffra, Rytelo, Tryngolza, Voranigo, Vyloy, Xolremdi, Zevtera, Ziihera



CDER designated 22 of the 50 novel drugs (44%) as fast track.

Breakthrough Therapy

CDER designated 18 of the 50 novel drugs (36%) in 2024 as breakthrough therapies. A breakthrough therapy designation includes all the fast track program features and offers intensive FDA guidance during drug development, including involvement from senior managers.

Drugs designated with breakthrough therapy status were:

Anktiva, Bizengri, Crenessity, Imdelltra, Iqirvo, Itovebi, Kisunla, Livdelzi, Miplyffa, Nemluvio, Ojemda, Revuforj, Rezdiffra, Tryngolza, Voranigo, Voydeya, Winrevair, Ziihera

Priority Review

In 2024, 28 of the 50 novel drugs approved (56%) were designated priority review. A drug receives a priority review if CDER determines that the drug treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions. Generally speaking, a priority review application is one on which CDER sets a goal to take action within 6 months of filing (compared to a target date of 10 months under standard review).

(In some instances, sponsors may redeem a priority review voucher under CDER's priority review voucher program. Such drugs are not included in the list below, and do not meet priority review criteria.)

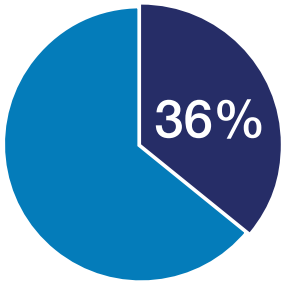
Drugs designated Priority Review were:

Alhemo, Aqneursa, Bizengri, Crenessity, Duvyzat, Exblifep, Imdelltra, Iqirvo, Itovebi, Kisunla, Lazcluze, Livdelzi, Lumisight, Miplyffa, Nemluvio, Niktimvo, Ojemda, Orlynvah, Revuforj, Rezdiffra, Tryngolza, Voranigo, Vyloy, Winrevair, Xolremdi, Yorvipath, Zevtera, Ziihera

Accelerated Approval

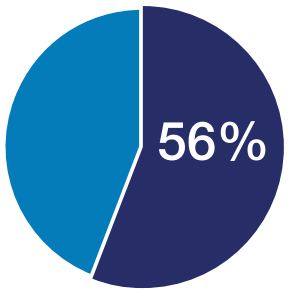
CDER approved 7 of the 50 novel drugs (14%) in 2024 under the accelerated approval pathway. This program works to bring certain drugs for unmet medical needs to market on a faster timeline than would be possible following the traditional approval pathway. Accelerated approval may be an option for a new drug intended to treat a serious condition that offers a meaningful advantage over available therapies. For drugs eligible to follow the accelerated approval pathway, a determination of safety and effectiveness will be made based not on measures of direct clinical benefit, but rather on one of two alternative endpoints: (1) a surrogate endpoint that is reasonably likely to predict clinical benefit; or (2) an intermediate clinical endpoint that is reasonably likely to predict clinical benefit. Use of such endpoints may enable the drug to be studied for a shorter duration than would be typical for a traditional approval, and to receive accelerated approval based on these findings. Importantly, however, for products approved under the accelerated approval pathway, FDA requires post-approval studies designed to confirm clinical benefit, and, among other things, may withdraw, using expedited procedures, an accelerated approval product from the market for failure to confirm clinical benefit.

Breakthrough Therapy



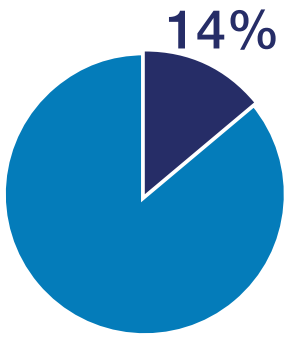
CDER identified 18 of the 50 novel drugs (36%) approved in 2024 as breakthrough therapies.

Priority Review



28 of the 50 drugs (56%) approved in 2024 were designated as priority review.

Accelerated Approval



CDER identified 7 of the 50 novel drugs (14%) as accelerated approvals.

The novel drugs approved via Accelerated Approval were:

Bizengri, Imdelltra, Iqirvo, Livdelzi, Ojemda, Rezdiffra, Ziihera

Overall Use of Expedited Development and Review Methods

33 of the 50 novel drug approvals of 2024 (66%) used one or more expedited programs, specifically fast track designation, breakthrough therapy designation, priority review designation, or accelerated approval.

Novel drugs approved in 2024 that used at least one expedited program were:

Alhemo, Anktiva, Aqneursa, Bizengri, Crenessity, Duvyzat, Ebglyss, Exblifep, Imdelltra, Iqirvo, Itovebi, Kisunla, Lazcluze, Leqselvi, Livdelzi, Lumisight, Miplyffa, Nemluvio, Niktimvo, Ojemda, Orlynvah, Revuforj, Rezdiffra, Rytelo, Tryngolza, Voranigo, Voydeya, Vyloy, Winrevair, Xolremdi, Yorvipath, Zevtera, Ziihera

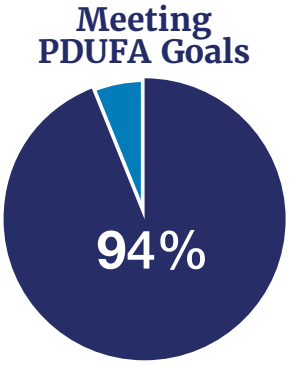
CDER used at least one expedited program to speed approval of 66% of all novel drugs approved in 2024.

Predictability: Meeting PDUFA Goals

Under PDUFA, industry is assessed user fees that provide resources to CDER to expand capabilities for review activities. With PDUFA, applications are reviewed targeting specific timeframes. Throughout 2024, CDER met or exceeded the PDUFA goal date for taking action on 94% (47 of 50) of the novel drugs approved.

Novel drugs approved in 2024 on or before their PDUFA goal dates were:

Alhemo, Alyftrek, Anktiva, Aqneursa, Attruby, Bizengri, Cobenfy, Crenessity, Duvyzat, Ebglyss, Ensacove, Exblifep, Flyrcado, Hympavzi, Imdelltra, Iomervu, Iqirvo, Itovebi, Lazcluze, Leqselvi, Letybo, Livdelzi, Miplyffa, Nemluvio, Niktimvo, Ohtuvayre, Ojemda, Orlynvah, Piasky, Rapiblyk, Revuforj, Rezdiffra, Rytelo, Sofdra, Tryngolza, Tryvio, Unloxcyt, Vafseo, Voranigo, Voydeya, Vyloy, Winrevair, Xolremdi, Yorvipath, Zelsuvmi, Zevtera, Ziihera



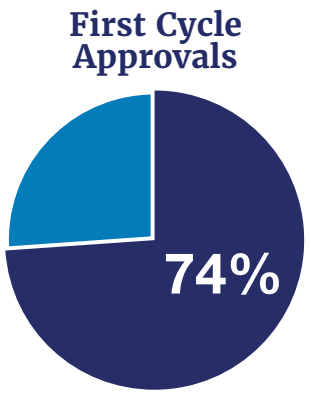
In 2024, 47 out of the 50 novel drugs (94%) were approved on or before their PDUFA goal date.

Access: First Cycle Approvals and First in U.S. Approvals

CDER approved 37 of the 50 novel drugs of 2024 (74%) on the “first cycle” of review. This high percentage is in part reflective of the extent to which CDER staff provide clarity to drug developers on the necessary study design elements and other data needed in the drug application to support a full and comprehensive drug assessment.

Novel drugs approved in 2024 on the first cycle were:

Alyftrek, Aqneursa, Attruby, Bizengri, Cobenfy, Crenessity, Duvyzat, Ensacove, Exblifep, Flyrcado, Hympavzi, Imdelltra, Iomervu, Iqirvo, Itovebi, Lazcluze, Leqselvi, Livdelzi, Lumisight, Nemluvio, Niktimvo, Ohtuvayre, Ojemda, Piasky, Revuforj, Rezdiffra, Rytelo, Tevimbra, Tryngolza, Tryvio, Voranigo, Voydeya, Winrevair, Xolremdi, Zelsuvmi, Zevtera, Ziihera



In 2024, CDER approved 37 of the 50 novel drugs (74%) on the first cycle.

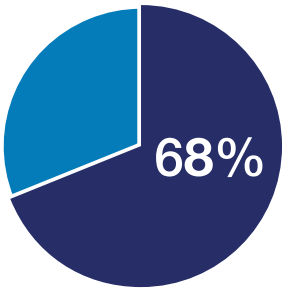
Approval in the U.S. Before Other Countries

34 of the 50 (68%) novel drugs approved in 2024 were approved in the U.S. before any other country.

Novel drugs of 2024 approved first in the U.S. were:

Alyftrek, Anktiva, Aqneursa, Attruby, Bizengri, Cobenfy, Crenessity, Duvyzat, Exblifep, Flyrcado, Hympavzi, Imdelltra, Iqirvo, Itovebi, Kisunla, Leqselvi, Livdelzi, Lumisight, Miplyffa, Niktimvo, Ohtuvayre, Ojemda, Orlynvah, Revuforj, Rezdiffra, Rytelo, Tryngolza, Tryvio, Unloxcyt, Voranigo, Winrevair, Xolremdi, Zelsuvmi, Ziihera

First in the U.S.



34 of 50 novel drugs (68%) approved in 2024 were first approved in the U.S.

New Uses of Approved Drugs

After CDER approves a new treatment, a drug sponsor may generate new data about the product that suggests an additional use. The drug sponsor may then submit an application to modify or expand the use of an approved drug based on this new data.

The products below are some 2024 CDER approvals for new uses or indications of an approved drug:

- **Alecensa (alectinib)** capsules were first approved in 2015 to treat anaplastic lymphoma kinase (ALK)-positive, metastatic NSCLC in adults who have progressed on or are intolerant to crizotinib. Alecensa was approved in 2024 as an adjuvant treatment (a cancer treatment given after the primary treatment) in adults following tumor resection of ALK-NSCLC. ALK-NSCLC is caused by a gene fusion (joining of two genes together) that produces an abnormal ALK protein that causes cancer cells to grow and spread.
- **Benlysta (belimumab)** for intravenous injection was first approved in 2019 to treat pediatric patients 5 years of age and older with active, autoantibody-positive, systemic lupus erythematosus who are receiving standard therapy. In 2024, Benlysta was approved as a subcutaneous autoinjector formulation for pediatric patients 5 years and older, allowing children to receive treatment at home. Systemic lupus erythematosus is a chronic autoimmune disease that causes the body's immune system to attack its tissues and organs.
- **Darzalex Faspro (daratumumab and hyaluronidase-fihj)** subcutaneous injection was originally approved in 2020 to treat multiple myeloma. In 2024, CDER approved Darzalex Faspro, in combination with bortezomib, lenalidomide, and dexamethasone, for induction and consolidation in patients newly diagnosed with multiple myeloma who are eligible for autologous stem cell transplant. Multiple myeloma is a rare cancer that occurs when abnormal plasma cells build up in the bone marrow and form tumors in the bones.

- **Enhertu (fam-trastuzumab deruxtecan-nxki)** for intravenous injection was first approved in 2019 to treat unresectable or metastatic HER2-positive breast cancer. In 2024, CDER approved Enhertu to treat adults with unresectable or metastatic HER2-positive (IHC 3+) solid tumors who have received systemic treatment and have no satisfactory alternative treatment options. HER2-positive solid tumors have high levels of the HER2 protein.
- **Epkinly (epcoritamab-bysp)** subcutaneous injection was initially approved in 2023 to treat relapsed or refractory diffuse large B-cell lymphoma. In 2024, CDER approved Epkinly to treat adults with relapsed or refractory follicular lymphoma after two or more lines of systemic therapy. Follicular lymphoma is a slow-growing cancer that may appear in lymph nodes, bone marrow, and other organs.
- **Fabhalta (iptacopan)** capsules were first approved in 2023 to treat paroxysmal nocturnal hemoglobinuria. In 2024, CDER approved Fabhalta for lowering proteinuria (protein in the urine) in adults with primary immunoglobulin A (IgA) nephropathy at risk of rapid disease progression. Primary immunoglobulin A nephropathy, also known as Berger disease, is a chronic kidney disease that occurs when IgA builds up in the kidneys.
- **Imfinzi (durvalumab)** intravenous injection was initially approved in 2017 to treat locally advanced or metastatic urothelial carcinoma. In 2024, CDER approved Imfinzi to treat patients with resectable NSCLC and no known epidermal growth factor receptor mutations or anaplastic lymphoma kinase (ALK) rearrangements. Resectable NSCLC is a stage of lung cancer that can be treated with surgery.
- **Livmarli (maralixibat)** oral solution was first approved in 2021 to treat cholestatic pruritus in patients with Alagille syndrome. In 2024, CDER approved Livmarli to treat progressive familial intrahepatic cholestasis, a rare genetic disorder that prevents the liver from secreting bile properly, which leads to liver disease and eventually liver failure.
- **Otezla (apremilast)** tablets were originally approved in 2014 to treat active psoriatic arthritis. In 2024, CDER approved Otezla to treat moderate-to-severe plaque psoriasis in adults. Plaque psoriasis is an autoimmune condition that causes thick skin patches (plaques).
- **Rybrevant (amivantamab-vmjw)** intravenous injection was first approved in 2021. In 2024, CDER approved Rybrevant as a first-line treatment for adults with locally advanced or metastatic NSCLC with EGFR exon 20 insertion mutations, as detected by an FDA-approved test. EGFR exon 20 insertion mutations can cause uncontrolled cell growth and are a biomarker in lung cancer.
- **Wegovy (semaglutide)** subcutaneous injection was originally approved in 2021. In 2024, CDER approved Wegovy to reduce the risk of major adverse cardiovascular events (cardiovascular death, non-fatal heart attack, or non-fatal stroke) in adults with established cardiovascular disease and either obesity or overweight.
- **Xolair (Omalizumab)** subcutaneous injection was initially approved in 2003 to treat adults and adolescents (12 years of age and older) with moderate-to-severe persistent asthma. In 2024, CDER approved Xolair to treat IgE-mediated food allergy in adult and pediatric patients aged 1 year and older for the reduction of allergic reactions (Type I), including anaphylaxis, that may occur with accidental exposure to one or more foods (to be used in conjunction with food allergen avoidance).
- **Zepbound (tirzepatide)** subcutaneous injection was first approved in 2023 for chronic weight management. In 2024, CDER approved Zepbound to treat obstructive sleep apnea. Obstructive sleep apnea causes breathing to stop and start repeatedly during sleep.

Approved Drugs Expanded for New Pediatric Populations

Section 505B of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (often referred to by the legislation that originally created it, the Pediatric Research Equity Act, or PREA) and section 505A of the FD&C Act (often referred to by the legislation that originally created it, the Best Pharmaceuticals for Children Act, or BPCA) give CDER the authority to require (PREA) or request (BPCA) pediatric studies under certain circumstances. These two laws have been largely responsible for the inclusion of pediatric information in the labeling for many drugs.

Upon drug approval, CDER may require pediatric studies of that drug under PREA. In response to that requirement, sponsors may submit new data to support the safe and effective use of the drug in the pediatric population studied. Sponsors submit this data in an application to expand the patient population. Under BPCA, sponsors may obtain additional marketing exclusivity for pediatric studies requested in a Pediatric Written Request.

The products below are examples of 2024 approvals for drugs expanded to include new pediatric populations:

- **Besponsa (inotuzumab ozogamicin)** for intravenous injection was initially approved in 2017 to treat adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia. Besponsa's patient population was expanded in 2024 to include pediatric patients 1 year and older. Acute lymphoblastic leukemia occurs when the bone marrow produces too many immature white blood cells called lymphocytes.
- **Blincyto (blinatumomab)** for intravenous injection was first approved in 2014 to treat CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia. In 2024, CDER expanded Blincyto's patient population to include pediatric patients 1 month and older.

- **Dovato (dolutegravir and lamivudine)** tablets were originally approved in 2019 to treat human immunodeficiency virus type 1 (HIV-1) infection in adults. Dovato's patient population was expanded in 2024 to include adolescents 12 years of age and over.
- **Dupixent (dupilumab)** subcutaneous injection was initially approved in 2017 for the treatment of adults with moderate-to-severe atopic dermatitis and was approved in 2022 for the treatment eosinophilic esophagitis in adults and pediatric patients 12 years of age and older. In 2024, CDER expanded the patient population for Dupixent to include pediatric patients aged 1 year and older who weigh at least 15 kg and have eosinophilic esophagitis. Eosinophilic esophagitis is a chronic allergic condition that causes inflammation in the esophagus.
- **Farxiga (dapagliflozin)** tablets and Xigduo XR (dapagliflozin and metformin hydrochloride) extended-release tablets were originally approved in 2014 to improve glucose control in adults with type 2 diabetes mellitus. In 2024, CDER expanded the patient populations to include pediatric patients aged 10 years and older with type 2 diabetes mellitus.
- **Lutathera (lutetium Lu 177 dotatate)** intravenous injection was originally approved in 2018 to treat somatostatin receptor-positive gastroenteropancreatic neuroendocrine tumors, including foregut, midgut, and hindgut neuroendocrine tumors in adults. In 2024, CDER expanded the patient population to include patients 12 years and older. Somatostatin receptor-positive gastroenteropancreatic neuroendocrine tumors are rare tumors that overexpress somatostatin receptors.
- **Veklury (remdesivir)** for intravenous injection was first approved in 2020 to treat coronavirus disease 2019 (COVID-19) requiring hospitalization in adults and pediatric patients 12 years of age and older. CDER expanded the patient population to include pediatric patients 1 month and older in 2022. In 2024, CDER expanded Veklury's patient population again to include patients from birth weighing at least 1.5 kg.

Biosimilar Approvals

The biosimilar pathway is an abbreviated approval pathway for biological products that are highly similar to and have no clinically meaningful differences in terms of safety, purity, and potency (safety and effectiveness) from an FDA-approved biological product, called a reference product. This pathway helps provide more treatment options, increase patient access, and potentially reduce the cost of therapies through competition.

In 2024, CDER approved 18 biosimilars for 8 reference products.

In 2024, CDER approved a total of 18 new biosimilars, including 8 for 3 reference products for which there were no previously approved biosimilars. Several of these biosimilars were approved as interchangeable biosimilars, which means that they may be substituted for the reference product at the pharmacy without the intervention of the prescriber, subject to state law.

- **Bkemv (eculizumab-aeeb)** intravenous injection is the first interchangeable biosimilar to the reference product Soliris (eculizumab), for the treatment of paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome. Atypical hemolytic uremic syndrome is a rare, progressive, and potentially life-threatening disease that causes abnormal blood clots in small blood vessels, primarily in the kidneys.
- **Jubbonti and Wyost (denosumab-bbdz)** subcutaneous injections are the first interchangeable biosimilars for reference products Prolia and Xgeva (denosumab), to treat osteoporosis and prevent bone events in cancer. Osteoporosis causes bones to become weak and more likely to break.
- **Opuviz (aflibercept-yszy)** and **Yesafili (aflibercept-jbvf)** intravitreal (in the eye) injections were approved as the first interchangeable biosimilars for the reference product Eylea (aflibercept), to treat macular degeneration and other eye conditions. Macular degeneration causes gradual vision loss in the center of the visual field.

In 2024, CDER also approved the notable changes to the following biosimilars:

- **Amjevita (adalimumab-atto)** subcutaneous injection was originally approved in 2016 to treat several inflammatory conditions, such as rheumatoid arthritis. In 2024, CDER approved it as an interchangeable biosimilar (reference product: Humira). Rheumatoid arthritis is a chronic autoimmune disease that causes joint pain, swelling, and stiffness.
- **Bkemv (eculizumab-aeeb)** intravenous injection was initially approved in 2024 to treat paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome. Later in 2024, CDER approved it to treat generalized myasthenia gravis (reference product: Soliris). Generalized myasthenia gravis is a chronic autoimmune disease that causes muscle weakness that can affect the entire body.
- **Epysqli (eculizumab-aagh)** intravenous injection was originally approved in 2024 to treat paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome. Later in 2024, CDER approved it to treat generalized myasthenia gravis (reference product: Soliris).
- **Eticovo (etanercept-ykro)** subcutaneous injection was first approved in 2019 to treat several inflammatory conditions, including psoriatic arthritis. In 2024, CDER approved it as an interchangeable biosimilar (reference product: Enbrel). Psoriatic arthritis is a chronic inflammatory disease that affects the joints and entheses, where tendons and ligaments attach to bones.
- **Hadlima (adalimumab-bwwd)** subcutaneous injection was initially approved in 2019 to treat inflammatory conditions such as juvenile idiopathic arthritis. In 2024, CDER approved it as an interchangeable biosimilar (reference product: Humira). Juvenile idiopathic arthritis is a chronic inflammatory disease that causes joint pain, swelling, and stiffness in children.
- **Hyrimoz (adalimumab-adaz)** subcutaneous injection was originally approved in 2018 to treat inflammatory conditions that included ulcerative colitis and ankylosing spondylitis. In 2024, CDER approved it as an interchangeable biosimilar (reference product: Humira). Ulcerative colitis is a chronic inflammatory bowel disease that causes inflammation and ulcers in the colon and rectum. Ankylosing spondylitis is a chronic inflammatory disease that causes inflammation in the joints and ligaments of the spine.
- **Tofidence (tocilizumab-bavi)** for intravenous injection was originally approved in 2023 to treat moderate-to-severe active rheumatoid arthritis in pediatrics and adults. In 2024, CDER approved it to treat hospitalized adults with COVID-19 who are receiving systemic corticosteroids and require supplemental oxygen, noninvasive or invasive mechanical ventilation, or extracorporeal membrane oxygenation (reference product: Actemra).

CDER has approved a total of 63 biosimilars for 17 different reference products since 2015. This includes at least 1 biosimilar for each of these top selling biologics in the U.S.: 10 biosimilars to Humira; 7 biosimilars to Stelara, 6 biosimilars to Herceptin and Neulasta; 5 biosimilars to Avastin and Eylea; 4 biosimilars to Neupogen and Remicade; 3 biosimilars to Rituxan; 2 biosimilars to Actemra, Enbrel, Lantus, Lucentis, and Soliris; and 1 biosimilar to Epogen/Procrit, Prolia/Xgeva, and Tysabri. Approval of multiple biosimilars for an approved reference product can enhance competition, which may lead to reduced costs for both patients and our health care system.

Other Important Approvals

New formulations of approved drugs can offer significant therapeutic advances. Similarly, new dosage forms (such as from a capsule to a chewable tablet for those unable to swallow pills) can help increase adherence, make sure patients take the proper dose, and improve quality of life for patients who must use the medication on a prolonged basis. Making a drug available as an over-the-counter product can also increase patient access to therapies. Below are examples of new formulations, new dosage forms, over-the-counter actions, and other notable approval actions of 2024:

- **Aurlumyn (iloprost)** intravenous injection is the first approved treatment for severe frostbite in adults to reduce the risk of digit (finger, toe) amputations. Iloprost, the active ingredient, had been previously approved to treat pulmonary arterial hypertension (high blood pressure in the arteries of the lungs, causing shortness of breath, dizziness, and tiredness).
- **Eohilia (budesonide)** oral suspension was approved as the first oral therapy to treat eosinophilic esophagitis in patients 11 years of age and older. Eohilia is approved as a 12-week course of treatment.
- **Lumify Preservative Free (brimonidine tartrate)** was approved as a new formulation to offer consumers the first preservative-free brimonidine-containing product to treat eye redness due to minor eye irritations.
- **Neffy (epinephrine)** nasal spray for emergency treatment of type I allergic reactions, including anaphylaxis, in patients who weigh 30 kg or greater. Neffy is the first non-injection epinephrine to treat anaphylaxis, a severe, life-threatening allergic reaction that can occur suddenly and rapidly.
- **Ocrevus Zunovo (ocrelizumab and hyaluronidase-ocsq)** subcutaneous injection was approved for the treatment of relapsing forms of multiple sclerosis (MS) and primary progressive MS in adults. Ocrevus Zunovo is a new dosage form of the

previously approved, intravenously administered product Ocrevus (ocrelizumab). MS is a chronic autoimmune disease that damages the protective coating around nerve cells in the brain and spinal cord.

- **Pivya (pivmecillinam)** tablets to treat females 18 years of age and older with uncomplicated urinary tract infections caused by susceptible isolates of *Escherichia coli, Proteus mirabilis* and *Staphylococcus saprophyticus.* Pivmecillinam is a derivative of pivmecillinam hydrochloride, an active ingredient previously approved for use in the U.S.
- **Prevymis (letermovir)** oral pellets for the prophylaxis of cytomegalovirus (CMV) infection and disease. This is a new dosage form of Prevymis that was previously only available as tablets or intravenous injection. Pellets are a small, spherical-shaped units that may be used for patients who cannot swallow tablets. CMV is a virus related to the herpes virus group of infections that is an incurable life-time infection.
- **Rezenopy (naloxone hydrochloride)** nasal spray is for the emergency treatment of known or suspected opioid overdose. Naloxone hydrochloride, the active ingredient, was previously approved, but Rezenopy contains a higher strength per dose than the other naloxone products available in the U.S.
- **Tecentriq Hybreza (atezolizumab and hyaluronidase-tqjs)** subcutaneous injection to treat NSCLC, hepatocellular carcinoma, melanoma, and alveolar soft part sarcoma. Tecentriq Hybreza is the first anti-PD-L1 antibody administered subcutaneously.
- **Voquezna (vonoprazan)** tablets for relief of heartburn associated with non-erosive gastroesophageal reflux disease in adults. The active ingredient for Voquezna is in a class of medications called potassium-competitive acid blockers. Voquezna is the first drug approved to use this active ingredient for this indication. Gastroesophageal reflux disease is a chronic condition that occurs when stomach contents leak back into the esophagus.
- **Zurnai (nalmefene)** intramuscular or subcutaneous injection for the emergency treatment of known or suspected opioid overdose induced by natural or synthetic opioids in patients aged 12 years and older, as manifested by respiratory and/or central nervous system depression. Zurnai is the first approved nalmefene hydrochloride auto-injector.

Please note that all drugs carry risks, and patients should review the drug labeling and consult with their health care professional to determine their preferred course of treatment.

Conclusion

From the time we receive a drug application — whether for a novel drug or a supplemental approval — our review process is a collaborative effort that involves scientific, regulatory, and policy experts from throughout CDER and sometimes other parts of FDA. For each application, we complete a very thorough and timely analysis of safety and effectiveness data, including a benefit-risk analysis that factors in the severity of the disease or condition, the currently available treatment options, and the intended patient population. If the therapy meets the standard for approval, we must reach agreement on the indication, labeling, safety issues, and other considerations.

We also gather external input from interested parties external to CDER and FDA. We often consult outside scientific experts, patients and patient advocates, industry representatives, academics, and other community members who are involved in drug development and review. Each of these parties has their unique expertise and perspective, and we consider their viewpoints. We take our regulatory decision-making seriously, because we know our decisions affect the health and well-being of patients and consumers nationwide.

Appendix A:
CDER's Novel Approvals of 2024
(in alphabetical order)

For information about vaccines, allergenic products, blood and blood products, cellular and gene therapy products, go to the 2024 Biologics License Application Approvals.

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA-approved use on approval date *(see Drugs@FDA for complete indication)*	Dosage Form
12/20/2024	Alhemo	concizumab-mtci	For routine prophylaxis of bleeding in hemophilia A and B.	Injection
12/20/2024	Alyftrek	vanzacaftor, tezacaftor, deutivacaftor	To treat cystic fibrosis.	Tablet
4/22/2024	Anktiva	nogapendekin alfa inbakicept-pmln	In combination with Bacillus Calmette-Guérin (BCG) to treat BCG-unresponsive nonmuscle invasive bladder cancer.	Intravesical solution
9/24/2024	Aqneursa	levacetylleucine	To treat neurological manifestations of Niemann-Pick disease type C.	Granules for oral suspension
11/29/2024	Attruby	acoramidis	To treat cardiomyopathy of wild-type or variant transthyretin-mediated amyloidosis.	Tablet
12/4/2024	Bizengri	zenocutuzumab-zbco	To treat non-small cell lung cancer and pancreatic adenocarcinoma.	Injection
9/26/2024	Cobenfy	xanomeline, trospium chloride	To treat schizophrenia.	Capsule
12/29/2024	Crenessity	crinecerfont	To treat classic congenital adrenal hyperplasia.	Capsule, Oral solution
3/21/2024	Duvyzat	givinostat	To treat Duchenne muscular dystrophy.	Oral suspension
9/13/2024	Ebglyss	lebrikizumab-lbkz	To treat moderate-to-severe atopic dermatitis.	Injection
12/28/2024	Ensacove	ensartinib	To treat NSCLC.	Capsule
2/22/2024	Exblifep	cefepime, enmetazobactam	To treat complicated urinary tract infections.	Injection
9/27/2024	Flyrcado	flurpiridaz F 18	To use with positron emission tomography (PET) myocardial perfusion imaging (MPI) to evaluate for myocardial ischemia and infarction.	Injection
10/11/2024	Hympavzi	marstacimab-hncq	To prevent or reduce the frequency of bleeding episodes with hemophilia A and B.	Injection
5/16/2024	Imdelltra	tarlatamab-dlle	To treat extensive stage small cell lung cancer.	Injection

Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA–approved use on approval date *(see Drugs@FDA for complete indication)*	Dosage Form
11/30/2024	Iomervu	iomeprol	To use with intra-arterial and intravenous procedures for diagnostic radiographic imaging.	Injection
6/10/2024	Iqirvo	elafibranor	In combination with ursodeoxycholic acid to treat primary biliary cholangitis.	Tablet
10/10/2024	Itovebi	inavolisib	In combination with palbociclib and fulvestrant to treat endocrine-resistant, PIK3CA-mutated, HR-positive, HER2-negative, locally advanced or metastatic breast cancer.	Tablet
7/2/2024	Kisunla	donanemab-azbt	To treat Alzheimer's disease.	Injection
8/19/2024	Lazcluze	lazertinib	In combination with amivantamab to treat locally advanced or metastatic NSCLC.	Tablet
7/25/2024	Leqselvi	deuruxolitinib	To treat severe alopecia areata.	Tablet
2/29/2024	Letybo	letibotulinumtoxinA-wlbg	To improve the appearance of moderate-to-severe glabellar lines.	Injection
8/14/2024	Livdelzi	seladelpar	In combination with ursodeoxycholic acid to treat primary biliary cholangitis.	Capsule
4/17/2024	Lumisight	pegulicianine	To use as an optical imaging agent for fluorescence imaging of breast cancer.	Injection
9/20/2024	Miplyffa	arimoclomol	In combination with miglustat to treat the neurological manifestations of Niemann-Pick disease type C.	Capsule
8/12/2024	Nemluvio	nemolizumab-ilto	To treat prurigo nodularis.	Injection
8/14/2024	Niktimvo	axatilimab-csfr	To treat chronic graft-versus-host disease.	Injection
6/26/2024	Ohtuvayre	ensifentrine	To treat chronic obstructive pulmonary disease (COPD).	Inhalation suspension
4/23/2024	Ojemda	tovorafenib	To treat pediatric low-grade glioma harboring an activating RAF alteration.	Tablet and Powder for oral suspension
10/25/2024	Orlynvah	sulopenem etzadroxil, probenecid	To treat uncomplicated urinary tract infections caused by the designated microorganisms *Escherichia coli, Klebsiella pneumoniae,* or *Proteus mirabilis* in adult women with limited or no alternative oral antibacterial treatment options.	Tablet

Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA–approved use on approval date *(see Drugs@FDA for complete indication)*	Dosage Form
6/20/2024	Piasky	crovalimab-akkz	To treat paroxysmal nocturnal hemoglobinuria.	Injection
11/29/2024	Rapiblyk	landiolol hydrochloride	To treat supraventricular tachycardia.	Injection
12/26/2024	Revuforj	revumenib	To treat relapsed or refractory acute leukemia with a KMT2A translocation.	Tablet
3/14/2024	Rezdiffra	resmetirom	To treat non-cirrhotic MASH (metabolic dysfunction- associated steatohepatitis) with moderate-to-advanced liver fibrosis (consistent with stages F2 to F3 fibrosis).	Tablet
6/6/2024	Rytelo	imetelstat	To treat low- to-intermediate-1 risk myelodysplastic syndromes.	Injection
6/18/2024	Sofdra	sofpironium bromide	To treat primary axillary hyperhidrosis.	Gel
3/13/2024	Tevimbra	tislelizumab-jsgr	To treat unresectable or metastatic esophageal squamous cell carcinoma.	Injection
12/19/2024	Tryngolza	olezarsen	To treat familial chylomicronemia syndrome.	Injection
3/19/2024	Tryvio	aprocitentan	In combination with other antihypertensive drugs to treat hypertension.	Tablet
12/28/2024	Unloxcyt	cosibelimab-ipdl	To treat metastatic or locally advanced cutaneous squamous cell carcinoma.	Injection
3/27/2024	Vafseo	vadadustat	To treat anemia due to chronic kidney disease.	Tablet
8/6/2024	Voranigo	vorasidenib	To treat Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation.	Tablet
3/29/2024	Voydeya	danicopan	To treat extravascular hemolysis associated with paroxysmal nocturnal hemoglobinuria.	Tablet
10/18/2024	Vyloy	zolbetuximab-clzb	In combination with fluoropyrimidine- and platinum-containing chemotherapy to treat locally advanced unresectable or metastatic HER2-negative gastric or gastroesophageal junction adenocarcinoma whose tumors claudin 18.2-positive.	Injection

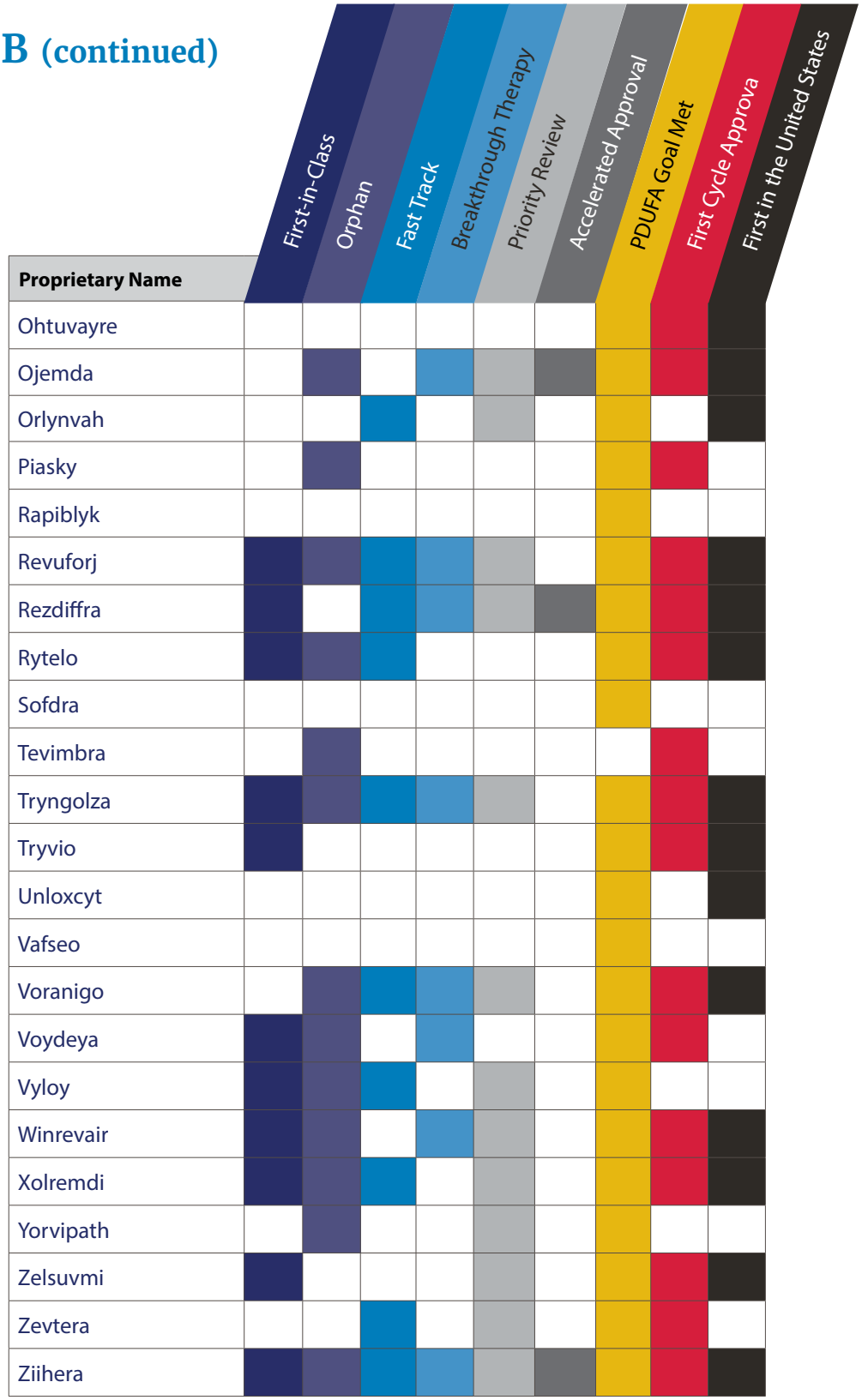
Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA–approved use on approval date *(see Drugs@FDA for complete indication)*	Dosage Form
3/26/2024	Winrevair	sotatercept-csrk	To treat pulmonary arterial hypertension (World Health Organization [WHO] Group 1).	Injection
4/26/2024	Xolremdi	mavorixafor	To treat WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis).	Capsule
8/9/2024	Yorvipath	palopegteriparatide	To treat hypoparathyroidism.	Injection
1/5/2024	Zelsuvmi	berdazimer	To treat molluscum contagiosum.	Gel
4/3/2024	Zevtera	ceftobiprole medocaril	To treat *Staphylococcus aureus* bloodstream infections (bacteremia), acute bacterial skin and skin structure infections, and community-acquired bacterial pneumonia.	Injection
11/20/2024	Ziihera	zanidatamab-hrii	To treat unresectable or metastatic HER2-positive (IHC 3+) biliary tract cancer.	Injection

Appendix B:
Novel Drug Designations
(in alphabetical order)

Proprietary Name	First-in-Class	Orphan	Fast Track	Breakthrough Therapy	Priority Review	Accelerated Approval	PDUFA Goal Met	First Cycle Approval	First in the United States
Alhemo		■			■		■		
Alyftrek		■					■	■	■
Anktiva	■		■	■			■		■
Aqneursa	■	■	■		■		■	■	■
Attruby		■					■	■	■
Bizengri	■	■	■	■	■	■	■	■	■
Cobenfy	■						■	■	■
Crenessity	■	■		■	■		■	■	■
Duvyzat	■	■	■		■		■	■	■
Ebglyss			■				■		
Ensacove							■	■	
Exblifep			■		■		■	■	■
Flyrcado							■	■	■
Hympavzi	■	■					■	■	■
Imdelltra	■	■		■	■	■	■	■	■
Iomervu							■	■	
Iqirvo	■	■		■		■	■	■	■
Itovebi				■	■		■	■	■
Kisunla			■	■				■	■
Lazcluze					■		■	■	■
Leqselvi			■		■		■	■	■
Letybo					■		■	■	■
Livdelzi		■		■	■	■	■	■	■
Lumisight	■		■					■	■
Miplyffa	■	■	■	■			■	■	■
Nemluvio	■			■	■		■	■	■
Niktimvo	■	■	■		■		■	■	■

Appendix B (continued)



Proprietary Name
Ohtuvayre
Ojemda
Orlynvah
Piasky
Rapiblyk
Revuforj
Rezdiffra
Rytelo
Sofdra
Tevimbra
Tryngolza
Tryvio
Unloxcyt
Vafseo
Voranigo
Voydeya
Vyloy
Winrevair
Xolremdi
Yorvipath
Zelsuvmi
Zevtera
Ziihera

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